

Muscular Dystrophy Association and MDA Venture Philanthropy



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Mission

The Muscular Dystrophy Association (MDA) is dedicated to finding treatments and cures for neuromuscular diseases – including muscular dystrophy, amyotrophic lateral sclerosis (ALS, or Lou Gehrig's disease), and related diseases – by funding worldwide research. The association also provides comprehensive healthcare and support services, advocacy, and education.

MDA Venture Philanthropy (MVP) is the Muscular Dystrophy Association's drug development program, which operates within MDA's Translational Research program. MVP is exclusively focused on identifying and funding the commercialization of treatments and cures for neuromuscular diseases.

Strategy/Approach

Founded in 1950, MDA provides research funding and support services for more than 40 neuromuscular diseases affecting more than 1 million Americans. The majority of diseases under MDA's umbrella are caused by genetic factors and result in progressive muscle weakness. To achieve its mission, MDA:

- **Funds worldwide research** – more than 300 projects around the world annually
- **Provides comprehensive health services** – through a national network of medical clinics and support services
- **Offers public and professional health education** – via meetings, materials, and online resources
- **Advocates on behalf of the neuromuscular disease community** – in matters of public policy and research
- **Coordinates community programs** – including MDA Art Collection, MDA summer camps, and myMDA.org, among others

In 2003, MDA started its **translational research program**, which is designed to move new drug targets into the clinic as rapidly and efficiently as possible. MVP is a component of the translational research program and leverages MDA's expertise in research into nerve and muscle biology to foster translation of findings into therapies.

Staff scientists track research developments from MDA's basic research program and from the scientific community as a whole and match the most promising ideas with drug development companies capable of developing such therapies. As a partner and not just a funder, MVP helps to cultivate follow-on investors, while its access to patients, experts, and research infrastructure can offset some costs of drug development.

Research Portfolio

MDA supports more research on neuromuscular diseases than any other private-sector organization in the world. MDA scientists are in the forefront of gene therapy research and have uncovered the genetic defects responsible for several forms of muscular dystrophy, Charcot-Marie-Tooth disease, a form of ALS, childhood spinal muscular atrophy, and several other neuromuscular conditions. Its main grant programs include:

- **Research Grants** – Designed to advance areas of scientific and medical knowledge that could improve understanding of the causes of neuromuscular diseases, or assist in developing strategies for their diagnosis and treatment.
 - *about \$100,000 per year for one to three years*
- **Development Grants** – Designed to expand the number of scientists conducting meritorious neuromuscular disease research from among investigators who may be part of a team in the laboratory of a senior investigator under whose guidance the researcher will be given flexibility to work independently or as part of a collaborative effort.
 - *Up to \$60,000/year for up to three years*

MDA's translational research program offers the following funding opportunities:

- **MDA Venture Philanthropy** – These targeted investments are reserved for projects for which the therapeutic of interest is unlikely to advance via traditional funding avenues due to the perceived risk of a small market, lack of a clear regulatory path, or lack of a new technology. They are focused on translational aspects of therapy development (e.g., optimization, scale-up, manufacturing, toxicology testing, and Phase I/II clinical trials), and awards are made based on the understanding that a therapeutic product will be brought to market by the awardee either during the tenure of the project or subsequent to the award.
 - *Range from \$500,000 to \$3 million over one to three years, depending on stage of research. Follow-on investment may be negotiated if all milestones are met.*
- **Clinical Research Training Grants** – Designed to provide promising young clinicians with the research training opportunities needed to become productive clinical investigators in neuromuscular disease research.
 - *\$90,000/year for two years*
- **Bridge to Industry Grant**—Awarded to young researchers as seed money to help launch scientific

programs of promising new neuromuscular disease researchers with an interest in drug development. The program must involve both academia and industry and have a drug development focus.

- \$60,000 per year for one to three years

Partnership Practices

MDA partners with academic scientists, nonprofits, and industry to achieve its goal of finding treatments and cures for neuromuscular diseases. MVP invests in small pharmaceutical and biotech companies and in academic scientists developing therapies. It funds projects from proof-of-principle studies through Phase II trials. Projects may include optimization, toxicology, manufacturing and scale-up, pre-IND, Phase I, or early Phase II trials.

MDA partners with biotech and pharmaceutical companies.

Companies may be public or private, in the United States or international. Examples include:

- **Asklepios BioPharmaceutical** – Awarded \$1.5 million to perform a Phase I study of a gene therapy technique that resulted in a muscle producing small amounts of dystrophin. Asklepios continued to develop this technique and is now progressing toward later-stage trials.
- **Glialogix** – Awarded \$268,000 to test a small molecule therapy previously tested for other diseases in the mouse model of ALS.
- **ReveraGen BioPharma** – Awarded \$1.5 million to develop a drug with the benefits of prednisone, but without the side effects, for Duchenne MD.
- **Repligen Corp.** – Awarded \$1,423,247 to get a novel spinal muscular atrophy (SMA) drug into a Phase I trial.

Financials

MDA is a 501(c)(3) nonprofit organization. The latest financial information available is from 2012.¹

Year ending 12/31/12:

- Revenue: \$152,115,051
- Assets: \$
- Grants: \$47,570,979
- Gifts received: \$149,557,236
- Expenditures: \$166,824,047

Key Accomplishments

- As of 2012, MDA had invested \$38.1 million in its research program, sponsored approximately 200 hospital-affiliated clinics and supported nearly 330 research projects around the world.
- In February 2013, MDA invested \$13.6 million in new research grants.
- The MDA Science Conference brought together 300 researchers, clinicians, representatives from biotech and pharmaceutical industries, and students.

- Sponsors approximately 200 hospital-affiliated clinics and supports more than 330 research projects around the world.
- The Food and Drug Administration gave a green light to a clinical trial of the experimental drug RG3039, developed to treat SMA by Repligen Corp., with a \$1.4 million grant from MDA.
- Thanks to MDA-funded research, disease-causing genetic mutations have been identified for most of the diseases in its program, and clinical trials are underway in Duchenne and Becker muscular dystrophies, SMA, ALS, and other diseases.
- Spends 77 cents of every dollar on its research and services programs. In 2010, patient and community services accounted for approximately 43 cents; research, 21 cents; and professional and public health education, 13 cents.

Leadership

The association is governed by a Board of Directors and counseled by a Translational Research Advisory Committee, a Medical Advisory Committee, and a Scientific Advisory Committee. MVP is managed by a small, Arizona-based staff.

- **President and CEO:** Steven Derks, sderks@mdausa.org
- **Executive Vice President and Chief Science & Medical Officer:** Valerie Cwik, MD
- **Director of Translational Research & MVP Portfolio Director:** Jane Larkindale, DPhil, jlarkindale@mdausa.org
- **Director of Clinical Research:** Heidi Pottinger, MA MPH
- **Executive Vice President and Chief Communications and Marketing Officer:** Steven G. Ford
- **Executive Vice President and Chief Development Officer:** Anne McNamara

¹ Information obtained from MDA Financial Statements, <http://mda.org/about/financials> March 2014.